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## *Fact Sheet*

## *Driving the Transformation to Predictive, Personalized, and Preemptive Medicine*

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### **Predicting Vulnerability to Disease and Response to Treatment.**

**High-tech Prediction of Alzheimer's Disease.** NIH-supported researchers used an inventive nanotechnology-based approach to diagnose early Alzheimer's disease (AD), which now can only be definitively determined by the examination of brain tissue after death. An ultrasensitive, nanoparticle-based strategy called barcode amplification was used to detect protein particles from Alzheimer's plaques and achieve a million-fold increase in sensitivity over the previously used test. By predicting which people are likely to suffer cognitive problems before they have symptoms, this diagnostic tool could ultimately lead to early treatment for this devastating disease, which afflicts about 3% of Americans over the age of 65 and almost half of those over 85. [Northwestern University](#); [RUMC-Chicago](#).

**Genetic Predisposition to Alcoholism Identified.** Scientists discovered that men carrying a certain genetic variation of a specific gene expressed in the nervous system were more likely to have problems with alcohol and anxiety. This new knowledge could enable prediction of who is at risk, help vulnerable people avoid alcoholism, and may also allow doctors to optimize treatment of these disorders. [NIDCR](#); [NIAAA](#); [CHBS Inc., Weatherford, OK](#); [Helsinki, Finland](#).

**Family History Helps Predict Which Women Will Develop Heart Disease.** Researchers found that women with no symptoms of heart disease, but with siblings with heart disease, had coronary artery calcium (40%) as well as severe atherosclerosis (6%). This finding indicates that an easy addition to the screening process—taking a family history—could help get at-risk women identified earlier and into preventative programs. [Altman, Stanford](#); [Flockhart, Indiana](#); [Giacomini, UCSF](#); [Krauss, Berkeley](#); [Licinio, UCLA](#); [McLeod, Washington U., St. Louis](#); [Nadkarni, Yale School of Medicine](#); [O'Connor, UCSD](#); [Ratain, University of Chicago](#); [Relling, St. Jude Children's Research Hospital](#); [Roden, Vanderbilt](#); [Weinshilboum, Mayo Clinic](#); [Weiss, Harvard Medical School](#).

### **Personalizing Prevention and Treatment Strategies.**

**Pharmacogenetics Leads the Way to Personalized Medicine.** NIH researchers used information derived from the human genome to identify genetic variations in individuals that alter their reaction to certain drugs. Their findings are being used to provide individually tailored drug dosing for a wide range of diseases including leukemia; breast, colorectal and lung cancers; heart arrhythmias; asthma; and depression. Importantly, information on individual genetic variation is also being used to identify individuals that may have severe adverse reactions to certain drugs.

**Combination Therapy Personalized to Fit Tumor-type Improves Breast Cancer Survival.** Two large clinical trials conducted by NIH Cancer Centers tested a combination therapy of trastuzumab and chemotherapy for patients with early-stage “HER-2 positive” breast cancer. Breast cancer patients are considered HER-2 positive if their cancer cells make too much of a protein called HER-2, which causes 1 in 3 tumors to grow faster and recur more often. Trastuzumab is a monoclonal antibody that inactivates the HER-2 protein. The trial was stopped and the results were made public before the clinical trials were completed because the studies showed that the addition of trastuzumab to chemotherapy significantly increased disease-free survival. For women with this type of aggressive breast cancer, trastuzumab reverses their prognosis from unfavorable to good. [Plantation, FL](#); [Mayo Clinic-Jacksonville, FL](#); [Mayo Clinic-Rochester, MN](#); [ACRI-Santa Monica, CA](#); [NCCC-Dartmouth](#); [NCI-Bethesda](#); [Kaiser, CA](#); [Oncology Associates-Cedar Rapids, IA](#); [U. Pittsburgh Cancer Institute](#); [WCCOP-Wichita, KS](#); [AHF-Canton, OH](#); [Genentech, CA](#).

**Personalizing Brain Tumor Treatment to Match Genetic Analysis.** Glioblastomas are the most common malignant brain tumors in adults, and they are notoriously difficult to treat successfully. NIH researchers determined that genetic analysis of glioblastomas can predict the tumor's sensitivity to specific drugs called Epidermal Growth Factor Receptor (EGFR) inhibitors. Specifically, glioblastomas with two specific genetic variations were 51 times more likely to shrink when treated with EGFR inhibitors, which translated into these patients going almost five times longer on average before their tumors progressed (243 days vs. 50 days). [UCLA](#); [UCSF](#); [UCSD](#); [Harvard Medical School](#); [MIT](#).

## **Preempting Disease Before Symptoms and Damage Occur.**

### **Effective Therapy for Osteoporosis Preempts Bone Loss.**

Osteoporosis, a disease in which bones become porous and subject to fracture, affects close to 10 million older people in the U.S. and is frequently detected only after a fracture occurs. One in two women and one in four men will have an osteoporosis-related fracture in her/his lifetime. A recent NIH study found that a sequential therapy consisting of a 1-year treatment with parathyroid hormone, which stimulates bone formation, followed by a 1-year treatment with alendronate, which slows breakdown of bone, is more effective in enhancing bone mineral density than using either of these treatments alone or simultaneously. [UCSF](#); [Columbia University](#); [Minneapolis Veterans Affairs Medical Center](#); [University of Minnesota, Minneapolis](#); [University of Pittsburgh Medical Center, Pittsburgh](#); [National Institute of Arthritis and Musculoskeletal and Skin Diseases \(NIAMS\)](#) ; [Maine Center for Osteoporosis Research, Bangor](#).

### **Human Papillomavirus (HPV) Vaccine Preempts Cervical Cancer.**

HPV infection causes virtually all cases of cervical cancer, the second most common cause of death from cancer in women. An investigational vaccine reduced HPV infection and precancerous disease by 90 percent. Several phase III trials testing the ability of the vaccine to protect against cervical cancer are underway. It is estimated that the successful completion of these trials and subsequent clinical dissemination of the vaccine could translate into preventing over 200,000 deaths per year worldwide from cervical cancer. [Brazil](#), [Finland](#), [Norway](#), [Sweden](#), [United States](#).

### **New Monoclonal Antibodies Neutralize Anthrax and**

**Preempt Lung Damage.** The anthrax attacks of 2001 re-focused attention on this deadly bacterium. Inhaled anthrax spores can lodge in the lungs, where they begin to grow and produce a deadly toxin. Even if all the anthrax bacteria are killed with antibiotics, the residual anthrax toxin can still be harmful or fatal. The powerful new monoclonal antibodies, developed by NIH-funded scientists, successfully bind to the toxin and prevent it from entering and killing cells. [National Institutes of Health \(NIH\)-Bethesda](#); [Bioqual-Rockville, MD](#).